To Manage Costs of Hemophilia, Patients Need More Than Clotting Factor

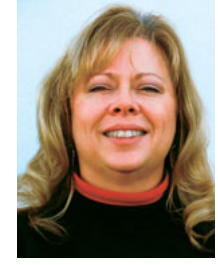
BY CRYSTAL S. BLANKENSHIP, PharmD
Accredo Health Services

With the cost of clotting factor exceeding $50,000 a year, careful management can save payers thousands of dollars per patient. The author writes about the role specialty pharmacy plays in cost management while helping patients achieve better clinical outcomes.

Improvements in medical care have allowed people with hemophilia to live longer, healthier lives, yet hemophilia remains one of the most costly and complex conditions to manage. Primary treatment involves infusing the patient with a clotting factor, costing tens of thousands of dollars annually. Given the complexity of the disorder, the cost of treatment, and the growing number of patients, it is critical for health plans and employers to manage hemophilia cases effectively. Specialty pharmacies can play a critical role as partners with the treating physician, family, and health plan by managing therapy, supplying pharmaceuticals, providing support to patients and families, and coordinating insurance coverage.

HEMOPHILIA POPULATION

According to the National Heart, Lung, and Blood Institute, about 18,000 Americans are diagnosed with hemophilia, and 400 children are born each year with this genetic disorder (NIH 2007). During the 1980s, clotting factor manufactured from human blood had inadequate viral inactivation, resulting in relatively high mortality rates caused by such bloodborne infections as HIV and hepatitis C. The impact of this can be seen today in that the hemophilia population is skewed toward the young; only 1 in 7 patients with hemophilia are 45 years of age or older (CDC 2008). Given that today's clotting factors are either recombinant or plasma-derived products that undergo extensive purification and viral inactivation, the life expectancy of people with hemophilia is closer to that of a healthy adult.



Crystal S. Blankenship, PharmD

Though mortality risks have declined, the disability risk for patients is still relatively high because the disorder is characterized by bleeding into muscles and joints, which can lead to chronic pain and joint disease. While it is common for people with hemophilia to bleed as a result of an injury, patients with severe cases of the disorder can bleed spontaneously. Even a seemingly minor injury requires intervention.

THERAPY MANAGEMENT PROCESS

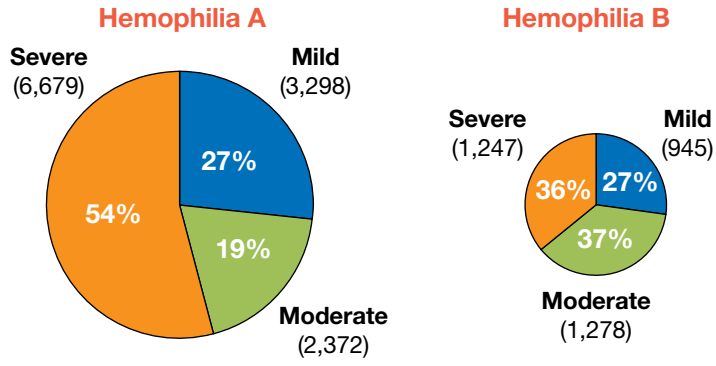
As with other chronic conditions that involve expensive treatments, hemophilia requires a high-touch approach to patient care. The therapy management process includes diagnosis, selection and evaluation of treatment, and psychosocial support for the patient. A physician must collaborate with nurses, pharmacists, social workers, and other members of the care team.

Specialty pharmacies also play a role as part of the treatment team, providing not just clotting factor, but also drug utilization management, patient education, and services that promote adherence to therapy and improved outcomes. Specialty pharmacies combine medical and pharmacy information into integrated data networks to give clinicians a holistic view of patient health that can guide the delivery of

Crystal S. Blankenship, PharmD, *has assisted patients as a national clinical therapy specialist for Hemophilia Health Services, an Accredo Health Subsidiary, for more than a year. Previously, she was a clinical pharmacist at Summit Medical Center, in Hermitage, Tenn., for six years. She obtained her PharmD from Auburn University.*

Figure
Hemophilia prevalence, severity in the United States

CDC Hemophilia Surveillance System data; cases recorded through Nov. 7, 2008



Source: CDC 2008

care. Through an assessment of each patient's lifestyle, environment, and social needs, a personalized care program is developed, including specialized training for the patient and caregivers.

Key components of a specialty pharmacy therapy management program include:

Diagnosis/stratification. The physician determines whether the patient has hemophilia A, hemophilia B, or another bleeding disorder, in addition to its severity. Hemophilia A patients, who compose the majority of the population, are deficient in clotting factor VIII, while hemophilia B patients are deficient in clotting factor IX.

In the general population, the clotting factor level in the blood can range from 50–200 percent of normal. People with *mild* hemophilia have clotting factor levels that are 5–50 percent of normal amounts; *moderate*, 1–5 percent; and *severe*, less than 1 percent. All hemophilia patients can experience massive bleeding from major trauma or during surgery, but people with severe hemophilia can also experience spontaneous bleeds in joints and muscles. According to the U.S. Centers for Disease Control and Prevention, about half of all hemophilia patients fall into the severe category (Figure).

Drug therapy. People who have hemophilia may be treated either on a regular basis with scheduled prophylaxis infusions or episodically at the time of a bleed. The U.S. Prophylaxis Study (Manco-Johnson 2007) showed that prophylaxis with recombinant factor VIII can prevent joint damage and the frequency of hemorrhage in some children with hemophilia — making it likely that prophylactic infusions will soon become more commonplace — but the physician ultimately determines the best type of treatment based on such patient factors as bleeding patterns, age, activity level, and response to therapy. Physicians may wish to consider collaborating with a specialty pharmacy that has expertise in managing hemophilia therapy, particularly if a patient has comorbidities such as hepatitis C and HIV, that require complex drug therapies.

The median cost of clotting factor products for a patient with hemophilia is in excess of $50,000 a year (Bohn 2004). Because of the high cost of treatment, patients should have their insurance coverage assessed and their lifetime health benefit caps evaluated. If spending levels are approaching their maximums, a specialty pharmacy can help to investigate alternative sources of funding, such as other health insurance options, charitable foundations, and/or government aid.

Social and educational needs. Clinicians at the specialty pharmacy assess the patient's lifestyle, psychosocial requirements, and the home environment. For example, a clinician can evaluate the ability of a parent or guardian to provide care to a pediatric patient; if the clinician should determine that a caregiver's knowledge about management of bleeding episodes is insufficient, training can be arranged. Similarly, the specialty pharmacy's clinical staff must acknowledge the challenges of coping with a chronic disease to help the patient's caregivers avoid burnout. Sometimes, support groups can assist patients and family members in satisfying their emotional needs. A patient's lifestyle also has a bearing on recommendations for care (see "Lifestyle: where care delivery becomes personalized," page 39).

Coordination and goal setting. The interdisciplinary clinical team at the hemophilia treatment center — including the hematologist, dentist, physical therapist, and other care providers — helps the person with hemophilia set goals. For example, dentists must work with patients and their hematologists to determine the best method to prevent bleeding during a proce-

Lifestyle: where care delivery becomes personalized

The patient's lifestyle affects everything from drug supplies needed to the actual course of treatment. Although administration of clotting factor by peripheral intravenous injection is optimal (Ewenstein 2004), the patient's physician may determine that implanting a central venous access device might be the best way to administer the clotting factor for patients with small veins or for children who require frequent injections. Specialty pharmacy personnel can provide training for the patient and family about care of the implanted device to help avoid complications.

Another lifestyle consideration is activity level. Protective gear, such as helmets and padding, are needed for toddlers, especially when they are learning to walk. As patients grow older, a hemophilia treatment center's clinical team — which usually includes a physical therapist — can recommend an individualized exercise program to strengthen and preserve muscle and joint function. Certainly, patients should avoid contact sports, such as football. Sports such as tennis, soccer, running, and baseball carry "medium risks" for bleeds because the physical, social, and psychological benefits often outweigh the risks (National Hemophilia Foundation 2006, Canadian Hemophilia Society 2006). During vacations and business travel, patients should bring supplies and equipment for transporting their clotting factor.

dure in a given person. If bleeds start to affect joint function, an orthopedic surgeon becomes a core member of this team. The specialty pharmacy should have a support structure that provides collaboration with treatment centers, as well as round-the-clock access to clinicians and home health care follow-up. The goal is clear: to prevent bleeds and establish procedures for administering the best course of treatment if a bleed occurs.

Appropriate intervention. A number of factors influence the treatment setting: site of the bleed, family knowledge and skill, and the knowledge of local medical personnel. People with hemophilia and their caregivers either can manage treatment at home with nursing assistance or proceed to a medical facility, such as a hemophilia treatment center or emergency room. Timely response and knowledge of what to do or how to seek help on the part of the patient or family member are critical when a bleed occurs.

The primary course of treatment is replacement of the deficient clotting factor. Ideally, infusion should occur within 3 hours of detection of a bleed to minimize the risk of long-term damage.

UTILIZATION MANAGEMENT

In patients who have hemophilia A, 1 unit of factor VIII/kg increases the clotting factor level in the blood by about 2 percent. The target levels of factor correction in patients with hemophilia B are the same as in patients with hemophilia A; however, the dose of the clotting factor should be higher because 1 unit of factor IX raises the circulating level by only 1 percent (Gioia 2004).

Clotting factor prescriptions are not written in a manner in which most of us are familiar, such as "10 mg once a day." Instead, clotting factor prescriptions are written as ranges. For example, one person may be prescribed a prophylactic dose of 2,000 units +/–10 percent, three times weekly, while another may be prescribed 1,500–1,700 units, three times weekly. Several variables contribute to the complexity of clotting factor dosing: the severity of the disorder, weight of the patient and manufacturing of clotting factor. Additionally, each patient may require varying doses for bleeds in different locations of the body. This is an important variable because bleeds in some locations — such as in the throat, eye, GI, pelvic muscle, or central nervous system — are more difficult to control (Gioia 2004).

Precise management of clotting factor can help to reduce waste and minimize healthcare costs. A utilization management program can help to ensure that patients are using clotting factor and supplies optimally. By monitoring bleeds, clotting factor inventory, utilization patterns, and use of adjunctive therapies, specialty pharmacists can assess treatment, facilitate communication between patients and their physicians, and help to optimize outcomes.

Some specialty pharmacies actively manage doses of clotting factor to achieve a near-zero variance from the number of prescribed units. They stock clotting factor

concentrates manufactured not in traditional dosage forms but in varying assays. Administering higher doses than prescribed does not harm patients, but overutilization provides no additional benefit and can increase annual treatment costs anywhere from 12 to 25 percent (Skinner 2000).

Managing dosage. The following examples illustrate the cost differential associated with small variances in clotting factor dosage. The availability of factor assays drives the dose that is dispensed when a prescription is written as a range. For a dose of 2,000 units +/–10 percent, 15 doses up to 2,200 units each could be dispensed and still meet the parameters of the prescription. If a small pharmacy only has a single assay of 1,095 units, then two vials would be dispensed to equal a dose of 2,190 units. A specialty pharmacy with an extensive inventory can match the target dose of 2,000 units more precisely. Assuming a clotting factor price of $1 per unit, then in this example, the specialty pharmacy saves the payer $190 on each dose, and to extend this example, a monthly use of 15 doses results in a savings of $2,850 per month.

Another example involves the prophylactic treatment of a 150-pound, 13-year-old boy, who has a target dose of 2,720 units of clotting factor three times a week. Assuming the same unit cost of $1 per unit, if the specialty pharmacy can maintain a 2 percent variance in the dosage rather than the 10 percent written into the prescription with more precise dose matching, the annual savings to the payer is $34,000 (Blankenship 2007).

Managing sources of coverage. The 13-year-old boy in the example above would reach the coverage limits of many commercial health plans after just a few years, after which he would require some other means to pay for the clotting factor. A specialty pharmacy can assist patients and their plan sponsors in managing the costs of hemophilia treatment across both medical and pharmacy benefit plans — so that fees are charged to the appropriate plan, thereby maximizing benefit coverage.

Assessment. A specialty pharmacist can review interventions and the amount of clotting factor used, then compare the results with goals for that patient's care. If a discrepancy is evident, the specialty pharmacist can alert the treating physician to changes in the patient's bleeding pattern, clotting factor usage, or lifestyle issues that may require changes to a treatment regimen. Beginning school and becoming an independent adult, for instance, are major changes in a patient's life that may require adjustments in therapy. Supply needs must be taken into account for these changes, such as having clotting factor available at school. It is important that the clinical team incorporates these changes into each patient's therapy management plan. Specialty pharmacies will report outcomes and cost savings to payers to demonstrate the value of their therapy management program.

SUMMARY

Managing hemophilia requires a coordinated effort by the patient, family members, the hemophilia treatment center, and the specialty pharmacy to minimize the impact of the disorder on the patient's life. Thanks to improvements in the treatment of hemophilia, most patients have no limits on activities. The specialty pharmacy's ability to combine therapeutic and utilization management for this high-cost condition, along with the ability to offer a holistic approach to patient care, can improve the effectiveness of hemophilia treatment — allowing patients with hemophilia to live normal lives with fewer bleeds, missed work days, and hospital visits.

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Disclosure

Crystal S. Blankenship, PharmD, is on the speaker's bureau of Pfizer.